



Prescription Drug User Fee Act (PDUFA) Reauthorization

FDA and Industry CBER Breakout Subgroup | Meeting Summary

October 13th, 2020 | 10:00am-12:00pm

Virtual Format (Zoom)

PURPOSE

To discuss FDA and industry CBER specific enhancement proposals.

PARTICIPANTS

FDA

Rachael Anatol CBER
Angela Granum CBER
Chris Joneckis (FDA Lead) CBER
Bharat Khanna CBER
Erik Laughner CBER
Carol Rehkopf CBER

Industry

E. Cartier Esham BIO
Brad Glasscock (Lead) BIO (BioMarin)
Robert Kowalski (Co-Lead) PhRMA (Novartis)
Heidi Marchand BIO (Gilead and Kite)
Lucy Vereshchagina PhRMA

The PDUFA VII CBER Breakout subgroup discussion focused on the Cell and Gene Therapy Program (CGTP) in CBER.

CBER Cell and Gene Therapy Program Proposal

CBER proposed an increase in staff to alleviate existing stress and strain on the CGT program. This increase in staffing would allow CBER to more adequately address existing work, get ahead of growth in the sector and ensure long-term sustainability. CBER would add capacity to increase the average time spent on CGT submissions, to account for novel development challenges, new regulatory requirements, guidance generation, and increased engagement with industry and stakeholders. CBER would appropriately resource all aspects of the program, including direct, indirect, and support functions, and modernize business, data and information technology. This effort would also make resources for the Regenerative Medicine Advanced Therapy (RMAT) program permanent.

CBER provided supportive data on the growth of IND volume, meeting requests, original BLA submissions, RMAT/BT designations, supplements, and adverse event reporting. CBER indicated that evolving science, novel manufacturing methods, and CMC challenges require a robust regulatory program with deep expertise with a need to make sure the regulatory science program is sufficient to support novel technologies.

Industry acknowledged the need to resource the CGT program and asked if FDA could provide some additional information on the proposal. CBER will provide data to facilitate the continued discussion.

There were no other substantive proposals, significant controversies, or differences of opinion discussed at this meeting.